

CIRM Tissue Collection for Neurodevelopmental Disabilities

Grant Award Details

CIRM Tissue Collection for Neurodevelopmental Disabilities

Grant Type: Tissue Collection for Disease Modeling

Grant Number: IT1-06611

Project Objective: To recruit a total of 550 tissue donors, and collect blood or skin, to ship to AllCells / Coriell for reprogramming by CDI.
450 donors will be patients with neurodevelopmental disorders (autism, intellectual disability, epilepsy, cerebral palsy) and family controls, 100 will be unrelated controls.

Investigator:

Name:	Joseph Gleeson
Institution:	University of California, San Diego
Type:	PI

Disease Focus: Neurological Disorders, Pediatrics

Cell Line Generation: iPS Cell

Award Value: \$830,426

Status: Closed

Progress Reports

Reporting Period: Year 1

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Reporting Period: Year 2

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Reporting Period: Year 3 NCE

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Grant Application Details

Application Title: CIRM Tissue Collection for Neurodevelopmental Disabilities

Public Abstract: Most children who go to the clinic with brain disorders have symptoms combining autism, cerebral palsy and epilepsy, suggesting underlying and shared mechanisms of brain dysfunction in these conditions. Such disorders affect 4-6% of the population with life-long disease, and account for about 10% of health care expenditures in the US. Genetic studies have pointed to frequent low-penetrant or low-frequency genetic alterations, but there is no clear way to use this information to make gene-specific diagnosis, to predict short- or long-term prognosis or to develop disease-specific therapy. We propose to recruit about 500 patients with these disorders mostly from our Children's Hospital, through a dedicated on-site collaborative approach. Extracting from existing medical records, taking advantage of years of experience in recruitment and stem cell generation, and already existing or planned whole exome or genome sequencing on most patients, we propose a safe, anonymous database linked to meaningful biological, medical, radiographic and genetic data. Because team members will be at the hospital, we can adjust future disease-specific recruitment goals depending upon scientific priorities, and re-contact patients if necessary. The clinical data, coupled with the proposed hiPSC lines, represents a platform for cell-based disease investigation and therapeutic discovery, with benefits to the children of California.

Statement of Benefit to California: This project can benefit Californians both in financial and non-financial terms. NeuroDevelopmental Disabilities (NDDs) affect 4-6% of Californians, create a huge disease burden estimated to account for 10% of California health care costs, and have no definitive treatments. Because we cannot study brain tissue directly, it is extraordinarily difficult to arrive at a specific diagnosis for affected children, so doctors are left ordering costly and low-yield tests, which limit prognostic information, counseling, prevention strategies, quality of life, and impede initiation of potentially beneficial therapies. Easily obtainable skin cells from Californians will be the basis of this project, so the study results will have maximal relevance to our own population. By combining "disease in a dish" platforms with cutting edge genomics, we can improve diagnosis and treatments for Californians and their families suffering from neurodevelopmental disorders. Additionally, this project, more than others, will help Californians financially because: 1) The ongoing evaluations of this group of patients utilizes medical diagnostics and genetic sequencing tools developed and manufactured in California, increasing our state revenues. 2) The strategy to develop "disease in a dish" projects centered on Neurodevelopmental Disabilities supports opportunities for ongoing efforts of California-based pharmaceutical and life sciences companies to leverage these discoveries for future therapies.

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